Factor Nine News

The Coalition for Hemophilia B Spring 2012

Topics in Hemophilia

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Fifth Annual Fundraising Dinner

The Coalition for Hemophilia B held its *Fifth Annual Fundraising Dinner* at the Waters Edge Restaurant with beautiful views of the Manhattan Skyline on Friday, March 16th, 2012. Approximately 200 people were in attendance, including the lotto families we flew in for the symposium, industry people and private donors. Monies raised will benefit the Coalition's educational programs and the William N. Drohan Scholarship Fund.

Cocktail hour included entertainment by the wonderfully talented pianist and saxophonists, William Gati and Ensemble, and dazzling Magician Henry Hu.

Dinner began with a warm welcome to our guests by Dr. David Clark, Chairman of The Coalition for Hemophilia B, followed by John Taylor who announced the William N. Drohan Scholarship winners. We were delighted to have Agnes Lubon, one of our scholarship winners in attendance.

After a delicious dinner, Dr. David Clark and his lovely little assistants announced the raffle prize winners. We continued the enjoyable evening dancing and enjoying the great company and beautiful views! Everyone had a good time indeed! We were delighted to see you all.

The Coalition for Hemophilia B sends a sincere thank you to all of our generous contributors. We hope you will join us again next year at our Sixth Annual Fundraising Dinner!


Starter
Entrée
Mushroom
Truffle

2012 Fundraising Dinner







EXIT













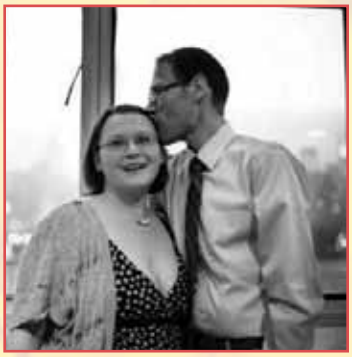








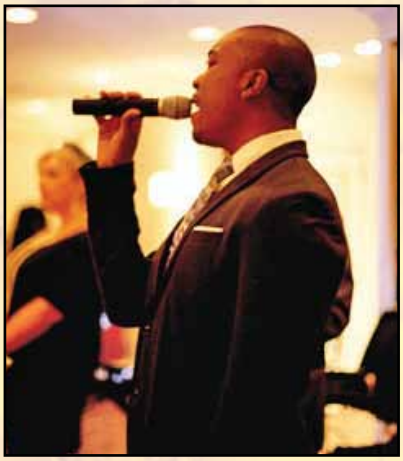

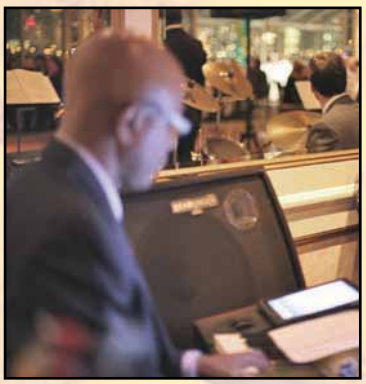
















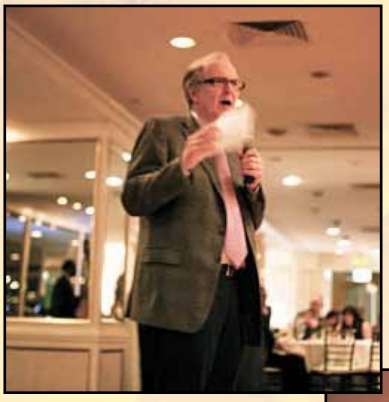





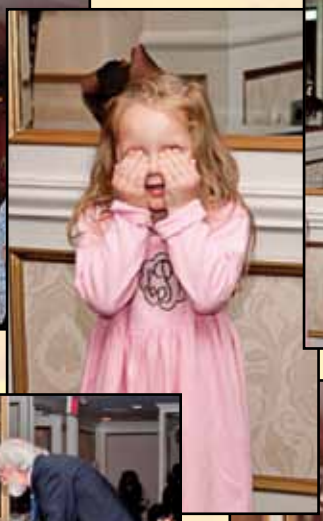







Meet Inspiration Biopharmaceuticals

We understand hemophilia

At Inspiration, we have made it our personal mission to make a difference in future treatment options available for people with hemophilia. The founding families of our company have been intimately impacted by hemophilia, as both families have sons who have hemophilia.

Our lead product candidate, currently in clinical trials, is a recombinant factor IX that is being developed for the treatment and prevention of bleeding in people with hemophilia B.

For more information, visit our website at www.inspirationbio.com

We are committed to:

- Broadening access to hemophilia care worldwide
- Improving treatment options for people with inhibitor complications

Inspiration
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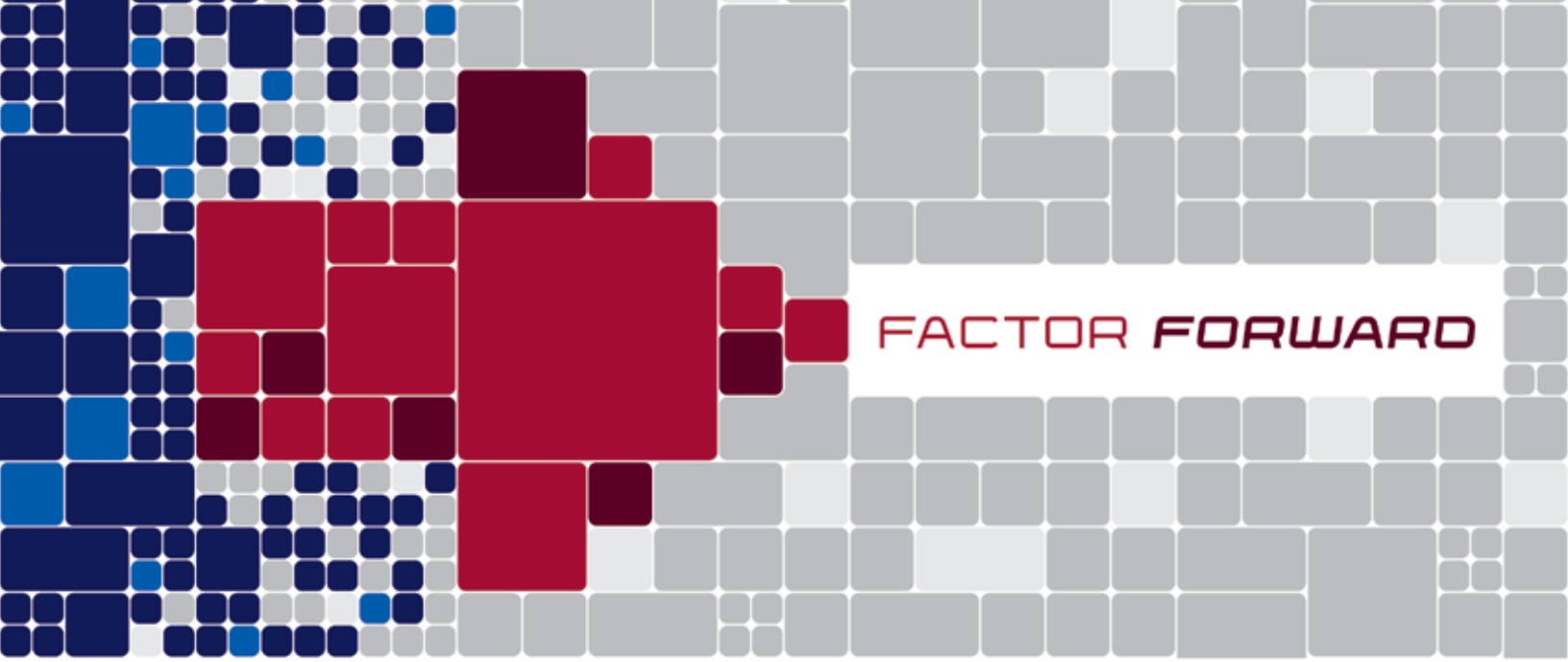

We are Biogen Idec Hemophilia,

and we're exploring ways to make clotting factors last longer.

Backed by more than 30 years of biotechnology leadership and meaningful therapies with global impact, Biogen Idec Hemophilia is blazing a new trail of scientific discovery toward long-lasting clotting factors. We're using forward-thinking technology to advance hemophilia research.

Biogen Idec Hemophilia is:

- A world-class team of scientists, clinicians, thought leaders, and visionaries with extensive experience in hemophilia
- Motivated by 1 goal: to make progress for the hemophilia community
- Driven to deliver one of the most anticipated therapeutic milestones in hemophilia—long-lasting clotting factors
- Passionate about R&D, which is led by Glenn Pierce, MD, PhD. Dr Pierce has dedicated more than 20 years to biotechnology R&D and served as President of the National Hemophilia Foundation
- Creating therapies, programs, and resources with the potential to change lives

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HEMOPHILIA

















6th Annual Symposium

The Coalition for Hemophilia B's Sixth Annual Symposium was held on Saturday, March 17th, 2012 at the New York Grand Hyatt Hotel. Pfizer Inc. generously funded the educational symposium.

The Symposium began with a warm welcome and opening comment by Wayne Cook, President of the Coalition. Our first speaker was Dr. Arthur Nienhuis of St. Jude Children's Research Hospital in Tennessee. His topic was on *Gene Therapy*. Dr. Nienhuis is a full member of the faculty in the Division of Experimental Hematology. We were grateful to him for flying in to share his news on gene therapy. Following Dr. Nienhuis was Dr. Christopher Walsh, Hematologist at Mt. Sinai School of Medicine in New York City. Dr. Walsh spoke about *What's New in Hemophilia*, a review of where we have been and where we are headed. Dr. Walsh's speech was very well received and followed with a multitude of questions from symposium participants.

Next on the agenda was Felix Garcia, who has hemophilia B and has been an active advocate for over nine years. His presentation *Hemophilia: A Family Affair* addressed the day-to-day emotional and social challenges faced by families dealing with hemophilia. The talk took you through a holistic view of the role, responsibilities and needs of each member of the family and addressed the topics of why it is important to be a proud member of the hemophilia community; how to feel good about the hemophilia in your family; the father-son relationship and how to play an active role in managing hemophilia in your family.

Afterward, everyone enjoyed a delicious lunch and visited the industry exhibit booths on display. Later in the afternoon, we heard from speakers Kevin Cosgrove, Executive Director of Pfizer's US Specialty Customers National and Specialty Accounts, and James Glonek, Director/Therapeutic Area Lead for the Specialty Care Business Unit and Government relations at Pfizer. Together Kevin and James updated us on Specialty Pharmacy-Market Evolution *What You Should Know*" and *The Impact to Patients, Physicians and the Factor Delivery System*. Both topics were well received and we thank them for coming out and educating our families on these very important and timely topics! After a quick break, Linda Pollhammer, Clinical Nurse Hemophilia Educator from Maryland with a background in infusion, home health care and support education discussed the *Life Stages of Hemophilia*. Linda did a wonderful job of taking us from infancy to older adults.

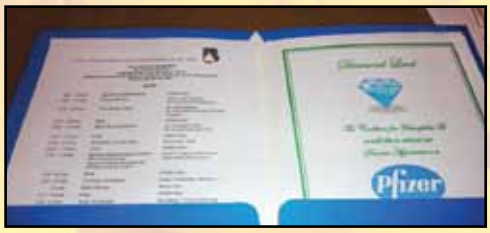
Later that afternoon we had breakout sessions, Peer Groups 12 and under moderated by Jill Lathrop, Peer Groups 13 and over moderated by Wayne Cook and Woman's Group. Following the sessions, we held our Factor Nine Family Meeting.

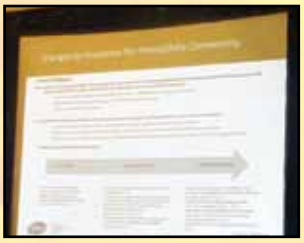
Babysitting was available on the premises for children under age five. The older children were escorted by several adult volunteers to attend the World Trade Center Memorial Museum followed by a true New York Pizza experience for lunch!

Happily, we had no causalities, but we did have an interesting twist to our event. There was another session going on the same floor and a young man was lost. He came to our booth thinking it was his group but we explained that this is for hemophilia B. Well, it turned out that this young man had hemophilia B, was from out of state and had never been to a meeting of any kind! We all welcomed him with open arms and he stayed for a while. We look forward to seeing him again at future meetings.

A big thank you to our sponsors, exhibitors and speakers for making our Annual Symposium a huge success!

6th Annual Symposium





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6th Annual Symposium Comments

My family had a wonderful time at the New York Symposium. The content the entire weekend was very informative and kept everyone's attention. This was our first trip to a Coalition for Hemophilia B event, and everyone could not be nicer or more helpful. It was a quick trip, but I feel that lasting friendships were made. My favorite session was the informal meeting the older guys had with the young boys, where they discussed bleeds and how the older boys dealt with them. I cannot provide that level of knowledge to my son, so that was something that I thought was very powerful and lasting. I hope to make this an annual trip for our family!
BP - Tennessee

We wanted to thank you for the wonderful time we had at the conference. We really enjoyed it. Felix Garcia was a great speaker. We enjoyed listening to him. A Big thanks to Wayne and the older men in the group that was just for boys. Thank you so much for showing my son how his arm can look if he doesn't alternate veins. It felt great to be surrounded by other people that can relate to our family. Love meeting everyone and being able to place names with faces. WE LOVED NYC.
GL - Texas

We really appreciated the opportunity to go to New York and meet all the wonderful members of the Coalition. This was a great time getting to meet and befriend all the people that we met. A special thanks for taking us in with open arms being a new family. We really loved getting to know crazy Aunt Jill, Felix, Kim, Wayne, and all the families that attended. This is an amazing support group, and friendships were created that will last a lifetime. Keep up all the good work and we look forward to seeing everyone again at the next gathering.
SS - Wyoming

It is so hard to encapsulate in a short paragraph everything attending the symposium means to our family. I'm sure if you asked each of us individually what our take away was, it would be different. The one constant for all of us is undoubtedly the opportunity to connect with others in our unique community. Having the opportunity to learn about the latest advancements in treatment for hemophilia B is what my husband and I so look forward to in coming to the symposium, but our kids look forward to fun times with their friends! The programming met a variety of needs and my entire family came away with new knowledge and friendships. We can't thank you enough for the wonderful experience! It was a weekend full of learning and laughter!
RS - Missouri

Words cannot explain our gratitude to the Coalition. Friday night we went out with the group to their Fundraising Dinners that had a beautiful nighttime water view of the Manhattan skyline. We met so many wonderful people that we had so much in common with. The next day we attended the conference where we learned about some of the treatments for hemophilia B that are coming up in the pipelines. It was a memorable experience. The many memories we made that week that would not have been possible without the help we received from The Coalition of Hemophilia B. Thank you all for a wonderful time. We hope to see you all next year!
MC - Florida

Our family would like to thank you for the opportunity provided for us to attend the symposium and the activities associated with the annual meeting. It was our first time attending any meeting regarding hemophilia B and the information provided was invaluable. I am comforted to know that research is ongoing for such a small number of individuals. The Hemophilia B community is to be commended for keeping their needs in the forefront. Thank you again.
AB - Virginia

Thanks again. We had a wonderful time at the Symposium. My oldest son was able to make some new friends and share his feelings about having hemophilia. Unfortunately, my youngest son still struggles with not "fitting in" and chooses to not discuss it. It was good for both of them to see how others handle their obstacles. It's been a whirlwind since we've been home, but again, thank you so much. I hope we are able to become more active in the hemophilia B community!
CC - Washington

Thank you for the opportunity to attend the Hemophilia B Symposium! This was the first time our family attended and we learned something new from every session. We especially found the *Update on Gene Therapy* with Dr. Arthur Nienhuis from St. Jude Children's Hospital and the session on *What's New in Hemophilia* with Dr. Christopher Walsh from Mt. Sinai Hospital particularly interesting. It was wonderful to meet other families with hemophilia B and spend time talking with them. My husband and son found the men and boys breakout session helpful in conveying to our son the importance of taking responsibility for your own health, and understanding the specifics of his type of hemophilia and factor product. We appreciate all the work you put into planning this event. It was a great experience and we hope to attend again in the future!
JB - Pennsylvania

BeneFix®

Coagulation Factor IX (Recombinant)

Room Temperature Storage

℞ only

Brief Summary

See package insert for full Prescribing Information. This product's label may have been updated. For further product information and current package insert, please visit www.Pfizer.com or call our medical communications department toll-free at 1-800-934-5556.

Please read this Patient Information carefully before using BeneFix and each time you get a refill. There may be new information. This brief summary does not take the place of talking with your doctor about your medical problems or your treatment.

What is BeneFix?

BeneFix is an injectable medicine that is used to help control and prevent bleeding in people with hemophilia B. Hemophilia B is also called congenital factor IX deficiency or Christmas disease.

BeneFix is **NOT** used to treat hemophilia A.

What should I tell my doctor before using BeneFix?

Tell your doctor and pharmacist about all of the medicines you take, including all prescription and non-prescription medicines, such as over-the-counter medicines, supplements, or herbal remedies.

Tell your doctor about all of your medical conditions, including if you:

- are pregnant or planning to become pregnant. It is not known if BeneFix may harm your unborn baby.
- are breastfeeding. It is not known if BeneFix passes into the milk and if it can harm your baby.

How should I infuse BeneFix?

The initial administrations of BeneFix should be administered under proper medical supervision, where proper medical care for severe allergic reactions could be provided.

See the step-by-step instructions for infusing in the complete patient labeling.

You should always follow the specific instructions given by your doctor. If you are unsure of the procedures, please call your doctor or pharmacist before using.

Call your doctor right away if bleeding is not controlled after using BeneFix.

Your doctor will prescribe the dose that you should take.

Your doctor may need to test your blood from time to time.

BeneFix should not be administered by continuous infusion.

What if I take too much BeneFix?

Call your doctor if you take too much BeneFix.

What are the possible side effects of BeneFix?

Allergic reactions may occur with BeneFix. Call your doctor or get emergency treatment right away if you have any of the following symptoms:

wheezing
difficulty breathing
chest tightness
turning blue (look at lips and gums)
fast heartbeat
swelling of the face
faintness
rash
hives

Your body can also make antibodies, called "inhibitors," against BeneFix, which may stop BeneFix from working properly.

Some common side effects of BeneFix are nausea, injection site reaction, injection site pain, headache, dizziness and rash.

BeneFix may increase the risk of thromboembolism (abnormal blood clots) in your body if you have risk factors for developing blood clots, including an indwelling venous catheter through which BeneFix is given by continuous infusion. There have been reports of severe blood clotting events, including life-threatening blood clots in critically ill neonates, while receiving continuous-infusion BeneFix through a central venous catheter. The safety and efficacy of BeneFix administration by continuous infusion have not been established.

These are not all the possible side effects of BeneFix.

Tell your doctor about any side effect that bothers you or that does not go away.

How should I store BeneFix?

DO NOT FREEZE BeneFix. BeneFix kit can be stored at room temperature (below 86°F) or under refrigeration. Store the diluent syringe at 36° to 86°F (2° to 30°C). Throw away any unused BeneFix and diluent after the expiration date indicated on the label.

Freezing should be avoided to prevent damage to the pre-filled diluent syringe.

Different storage conditions are described below.

Product labeled for Room Temperature Storage
Store at 2° to 30°C (36° to 86°F).
If you have the product kit labeled for room temperature storage, it can be stored at room temperature (below 30°C or 86°F) or in the refrigerator (2° to 8°C or 36° to 46°F).

Product labeled for Refrigerator Storage
Continuous refrigeration
[2° to 8°C (36° to 46°F)]
If you have the product labeled for storage in the refrigerator (2° to 8°C or 36° to 46°F) and you have not taken the kit out of the refrigerator, then the expiration date printed on the package still applies. You can store the product at room temperature (below 30°C or 86°F) for up to 6 months or until it has reached its expiration date, whichever comes first.

If you have taken the product kit labeled for storage in the refrigerator out of the refrigerator and stored it at room temperature (below 30°C or 86°), then use the product within 6 months from the time you took the product out of the refrigerator or until it has reached its expiration date, whichever comes first. If you cannot remember when you took it out of the refrigerator, then subtract one year (12 months) from the date that is printed on the end flap of the carton package. The date you get is your new expiration date. Throw away any product that has gone over the new expiration date.

BeneFix does not contain a preservative. After reconstituting BeneFix, you can store it at room temperature for up to 3 hours. If you have not used it in 3 hours, throw it away.

Do not use BeneFix if the reconstituted solution is not clear and colorless.

What else should I know about BeneFix?

Medicines are sometimes prescribed for purposes other than those listed here. Do not use BeneFix for a condition for which it was not prescribed. Do not share BeneFix with other people, even if they have the same symptoms that you have.

If you would like more information, talk to your doctor. You can ask your doctor for information about BeneFix that was written for healthcare professionals.

This brief summary is based on BeneFix® [Coagulation Factor IX (Recombinant)] Prescribing Information LAB-0464-8.0, revised November 2011.

Wyeth®

Manufactured by Wyeth Pharmaceuticals Inc.

Marketed by Pfizer Inc.

Printed in USA/November 2011

Pfizer

15+ YEARS* EXPERIENCE MATTERS

BeneFix®
Coagulation Factor IX (Recombinant)
Room Temperature Storage

The individual depicted is not a hemophilia patient.
For illustrative purposes only.

*BeneFix was approved February 11, 1997.

Please see brief summary of full Prescribing Information for BeneFix on reverse side.

You asked for 3000 IU in a single vial with the same 5-mL diluent. You got it.

3000 IU IS HERE

Let the infusion begin.

Visit the **NEW BeneFix.com** to learn more.

What Is BeneFix?

BeneFix® Coagulation Factor IX (Recombinant) is an injectable medicine that is used to help control and prevent bleeding in people with hemophilia B. Hemophilia B is also called congenital factor IX deficiency or Christmas disease.

BeneFix is **NOT** used to treat hemophilia A.

Important Safety Information for BeneFix

- BeneFix is contraindicated in patients who have manifested life-threatening, immediate hypersensitivity reactions, including anaphylaxis, to the product or its components, including hamster protein.
- Call your health care provider right away if your bleeding is not controlled after using BeneFix.
- Allergic reactions may occur with BeneFix. Call your health care provider or get emergency treatment right away if you have any of the following symptoms: wheezing, difficulty breathing, chest tightness, your lips and gums turning blue, fast heartbeat, facial swelling, faintness, rash or hives.
- Your body can make antibodies, called "inhibitors," which may interfere with the effectiveness of BeneFix.
- If you have risk factors for developing blood clots, such as a venous catheter through which BeneFix is given by continuous infusion, BeneFix may increase the risk of abnormal blood clots. The safety and efficacy of BeneFix administration by continuous infusion have not been established.
- Some common side effects of BeneFix are nausea, injection site reaction, injection site pain, headache, dizziness and rash.

You are encouraged to report negative side effects of prescription drugs to the FDA. Visit www.fda.gov/medwatch, or call 1-800-FDA-1088.

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ADHD in Boys with Hemophilia

Children with Attention Deficit Hyperactivity Disorder (ADHD), the most common childhood psychiatric disorder, tend to be inattentive, impulsive and over-active. A few studies have suggested that ADHD occurs at a higher rate in boys with hemophilia, but the evidence is not definitive. ADHD affects health and social adjustment, but it is highly treatable. Especially important for individuals with a bleeding disorder, ADHD is associated with a four times higher rate of physical injury than for children without ADHD.

ADHD occurs in about 3 - 5 % of school-age children, and is more common in boys than in girls. There is some evidence that it runs in families, but whether it has an actual genetic cause is unclear. It appears to begin early in life while the brain is developing. Imaging studies have shown definite differences in the brains of children with and without ADHD.

Studies of psycho-social disorders like ADHD can be difficult to conduct since the symptoms can't be measured as accurately as, for instance, the level of factor IX in the blood. Studies usually rely on large numbers of subjects and complex statistical analyses. Therefore, studying something like ADHD in the relatively-small hemophilia population is difficult. Only a few studies have been conducted, and although the results have suggested a statistically-significant higher incidence of ADHD, the evidence is not overwhelming.

The potential causes are also not clear. It is possible that there is a genetic component, but there is no apparent reason it should be associated with the genetic defect in hemophilia. Some wonder whether early undiagnosed bleeding in the brain or spinal cord could lead to ADHD. It could also be associated with the family's emotional stress in coping with a chronic disease.

In any case, it is one more thing to be aware of. Early identification and treatment can prevent many of the problems associated with ADHD. There should be no stigma associated with a psychological disorder like ADHD any more than for a physical disorder like hemophilia. Concerned parents should consult with their physician.

There are some good sources of information on the internet such as the NIH PubMed web site at http://www.ncbi.nlm.nih.gov/pubmedhealth/PMH0002518/.

Controversy in the Search for Hepatitis C Treatments

Hepatitis C is a liver disease that affects many people, including many older hemophilia patients. Plasma-derived clotting factors made before effective viral inactivation and screening methods were available in the late 1980s often transmitted the hepatitis C virus (HCV). Hepatitis C is a serious disease that can lead to cirrhosis and liver cancer. It is currently the leading cause of death in people with bleeding disorders.

Two new treatments were introduced last year (see the Spring/Summer issue of Factor IX News) that greatly improved hepatitis C treatment. However, both therapies still take months, are difficult for the patient to tolerate and are not always effective. Therefore, better treatments are needed, and several pharmaceutical companies are racing to develop them, partly because of the huge potential market.

Recent studies have shown that two experimental treatments, daclatasvir being developed by Bristol-Myers Squibb (BMS) and GS-7977 being developed by Gilead Sciences, are very effective when taken together. A collaboration between BMS and Gilead has been proposed, however, Gilead has reportedly delayed entering into the collaboration while it determines whether it can get similar results from GS-7977 in combination with one of its own experimental drugs. This potentially slows introduction of an effective treatment.

A group called *People with Bleeding Disorders and HCV* has started an effort to encourage Gilead to proceed with the collaboration with BMS to jointly develop the new drugs. They have drafted a letter, which is available online at https://docs.google.com/document/d/1wNiZYHtEp-HjgePYe6KxxyIaUvfwvZV0cZy89QPp17E/edit, that describes the situation in detail. The New York Times online article referenced in the letter also provides a good summary of the issues.

This is a good example of the economics and politics of drug development, which may seem to completely disregard consideration of the people who need a drug. It can be a frustrating situation for the patients involved, especially if they don't necessarily have time to wait for the outcome. However, one should also keep in mind that this is probably not the only treatment that will work. It may not actually be the best treatment in spite of the current evidence, and may not be the first available. Many drugs that seem effective in small scale studies don't pan out in larger trials. Also, the competition may have something better in the pipeline that we don't know about - much drug development is kept secret until it gets close to market.

If you are infected with HCV, don't despair. Support the movement to encourage the Gilead-BMS collaboration, if you are so inclined, but also remember that this is not the only path. Several effective new hepatitis C treatments will likely become available in the next few years.

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Antidepressants and Clotting

Hemophilia patients know that they should be careful about taking aspirin because of its effect on clotting, but many do not know that certain antidepressants can have a similar effect. Aspirin affects platelets, which are the smallest blood cells, and are part of the normal clotting mechanism of the blood. It turns out that some antidepressants may also inhibit the clotting activity of platelets.

Many people assume that the danger in hemophilia is that someone could bleed to death from a small cut, but we know that's not really true. The reason that doesn't happen is because of platelets. An injury to a blood vessel activates the coagulation system to produce a clot, which doesn't occur correctly in people with hemophilia. However, the chemical signals from a damaged blood vessel also activate platelets.

Activated platelets stick together (aggregate) to form a plug that reinforces the clot and seals the blood vessel. Activated platelets also release a number of growth factors, small proteins that control the healing process. Even with a defective clotting mechanism, the platelet plug still forms and stops the bleeding - it just takes longer and the clot is not as strong.

Aspirin, among its many effects, inhibits production of thromboxane, a protein that makes activated platelets sticky, so it inhibits the platelet portion of the clotting mechanism. Serotonin is another chemical compound that is involved in platelet aggregation, and it is also a neurotransmitter that affects our feelings of well being. Neither role of serotonin is completely understood, but researchers have found that drugs that inhibit the uptake of serotonin by brain cells can be effective treatments for depressed patients. These include the older tricyclic antidepressants and the newer Selective Serotonin Reuptake Inhibitors (SSRIs, such as Prozac, Paxil and Zoloft) and Serotonin Norepinephrine Reuptake Inhibitors (SNRIs, such as Effexor and Cymbalta).

A number of studies have shown that patients on SSRIs and SNRIs tend to have a higher risk of bleeding. The studies did not include hemophilia patients, and the effects were not always large. The risk of bleeding appears to increase with the strength of serotonin reuptake inhibition of the drug.

The idea is that the SSRIs and SNRIs may also inhibit uptake of serotonin by platelets and thus affect platelet aggregation, the formation of a platelet plug. Platelets take up serotonin from the bloodstream, since they can't make their own. Thus a drug that affects the ability of a brain cell to take up serotonin may also affect the ability of platelets to take up serotonin, and thus to aggregate and form a clot.

So, should hemophilia patients avoid antidepressants? Not necessarily. Not all SSRIs and SNRIs have a large effect on clotting, and there are other antidepressants that work by other mechanisms. Plus, depression is not just a matter of feeling low. It can have significant effects on overall health, including increasing the risk of heart disease. A hemophilia patient should work with his or her doctor to find the right treatments that take into account both their depression and their hemophilia.

A Message From Kim...

Hello All!

Over the past several months we have received many requests for assistance from families in our community. We believe it takes a village, even $5.00 will make a tremendous difference in the quality of life for people with hemophilia in need. We thank you for all your love, kindness and generosity to help families in need! And to make things easier, we have exciting news! We now have a PayPal account to make it easier to make donations! Just visit our website coalitionforhemophiliab.org and click on donate, which will bring you directly to our PayPal site or go to PayPal and use our email (hemob@ix.netcom.com) address to donate.

Thank you! Thank you! ***Thank you!!!***

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1500 IU FIX Range	10 mL

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Treatment News

Much has happened since our last review of new products and treatments for hemophilia B in the Fall 2011 issue of Factor Nine News. Here's a summary of some of the latest news. Note the increased interest in gene therapy products and a unique gene therapy treatment for HIV/AIDS.

There has also been a lot of activity in development of treatments for inhibitor patients that will be covered in a future issue. These are mainly improved versions of activated factor VII, the active ingredient in NovoSeven.

Conventional Factor IX Products

- Baxter expects to file a BLA for their BAX326 recombinant factor IX by the end of 2012.
- Bayer is developing a recombinant factor IX product, but no details are available.
- Inspiration Biopharmaceuticals filed a Biologics License Application (BLA) with the FDA for U.S. licensure of their recombinant Factor IX product. Inspiration also recently added several new executives and moved to a permanent headquarters in Cambridge, MA after years as a virtual company.

Longer-Acting Factor IX Products

- CSL-Behring announced the results of a Phase I study of a recombinant factor IX that is linked to albumin to increase its half-life in circulation. The product, CSL654, was well tolerated in all patients, produced no inhibitor reactions and exhibited a half-life five times longer than current FIX products. CSL also received Orphan Drug Designation in the U.S. for the product. Orphan Drug Designation is for treatments that affect fewer than 200,000 people and provides tax advantages and marketing exclusivity for the first product licensed of a given type.
- Pro Bono Bio Group and PolyTherics are developing TheraPEG™ Factor IX, a longer-acting PEGylated product. They have completed preclinical pharmacology studies and are now developing a large-scale manufacturing process to produce material for clinical studies. The two companies are also collaborating on TheraPEG™ versions of factor VIII for hemophilia A and factor VIIa for inhibitor treatment.

Alternative Treatments for Hemophilia

- Alnylam Pharmaceuticals has reported good clinical results for use of their RNA interference (RNAi) method for treatment of two non-hemophilia diseases. RNAi is a technique that can reduce the amounts of a targeted protein produced by the body. They have recently begun a program to look at reducing levels of Protein C, a natural anticoagulant in the blood. By lowering the amount of Protein C, they hope to increase clotting activity.

Gene Therapy

Possibly because of the encouraging results obtained at the end of 2011 in the gene therapy study by St. Jude's Children's Research Hospital and University College London (see Factor IX News, Fall 2011), there has been much activity on the gene therapy front.

- Amsterdam Molecular Therapeutics, a collaborator in the St. Jude's-UCL studies, has received Orphan Drug Designation in both the U.S. and Europe for their gene therapy treatment. They were recently acquired by uniQure, a newly-formed private company.
- Avalanche Biotechnologies and Lonza are collaborating on processes for manufacture of the adeno-associated virus (AAV) vectors that have become one of the most promising methods for gene therapy. They plan to license their technology to other companies.
- Baxter and Chatham Therapeutics have started a collaboration to develop a gene therapy treatment for hemophilia B based on Chatham's proprietary technology. Chatham's Biological Nano Particles™ are reported to be an advanced AAV-based method for delivering genes to cells. Chatham, which is an affiliate of Asklepios BioPharmaceutical, is also collaborating with ReGenX to use some of ReGenX's AAV technology. Asklepios has collaborated with Bayer for development of gene therapy treatments for hemophilia B, but the status of that collaboration is unknown.
- Sangamo BioSciences and Shire are collaborating on gene therapy for hemophilia B using Sangamo's zinc finger technology. Instead of replacing genes, zinc finger enzymes can be used to edit existing genes to correct defects. They believe that editing existing genes is a safer alternative that also preserves the natural regulation of the gene. They have seen promising results in animal studies of hemophilia B.

Gene Therapy for AIDS

Finally, a group at the University of Pennsylvania has used gene therapy to treat T cells in HIV-positive patients. T cells are a type white blood cell that fights infections and tumors. The T cells were modified to be able to better recognize HIV, the AIDS virus. This is a long-term study in which 43 HIV patients were given some of their own T cells that had been modified in the laboratory and then re-infused. The infusions took place between 1998 and 2002, and the patients have been followed since then. All of the patients are healthy and have significantly-reduced HIV levels, comparable to the effect of treatment with anti-HIV drugs, but without the side effects of the drugs. None have experienced any significant adverse reactions from the T cell treatment.

Eric Lowe
with his father

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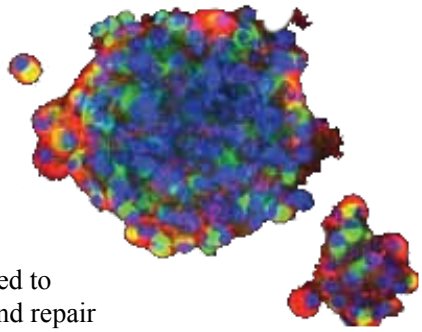
Hemophilia B Stem Cell Lines for Research

The University of Michigan (UM) recently announced that it had developed several new embryonic stem cell lines including two that contain genetic defects for hemophilia B. The cell lines will give researchers new tools to better understand the disease and for potentially developing and testing new treatments.

Stem cells are cells that have not transformed into any specific type of cell, a process called differentiation. They are useful because they have the potential, under the right conditions, to differentiate into many different kinds of cells, such as liver, skin or muscle cells. There are two main types of stem cells, embryonic and adult stem cells.

Embryonic stem cells are the cells that form when a fertilized egg first starts dividing to produce an organism. Human embryonic stem cells have been the subject of controversy because they come from human embryos. Since human embryos have the potential to grow into human beings, any other use is considered by some people as equivalent to abortion. Except in a few states like California and Michigan that passed their own laws, research on embryonic stem cells has been highly restricted by the federal government until recently.

Embryonic stem cells are usually obtained as a result of in vitro fertilization (IVF) procedures for couples who have difficulty conceiving children. IVF procedures often produce more embryos that can be used, and the extra embryos are usually discarded. The UM cell lines were derived from five-day-old embryos that had been screened and found to contain various genetic defects, including hemophilia. The couples from whom the embryos were created donated them to the university rather than having them discarded.



Because of the controversy and the federal restrictions on embryonic stem cells, many researchers have instead focused on adult stem cells. Our bodies are full of adult stem cells, which are used to replace worn out cells and repair injuries. Researchers have developed techniques with which adult stem cells can be used in place of embryonic stem cells for many, but not all uses.

A cell line is a group of cells that is maintained in a laboratory. The cells are grown in laboratory containers and fed a solution (media) that contains the nutrients that the cells need to live and grow. This is called in vitro tissue culture, where the Latin in vitro means "in glass", although today most of the tissue culture containers are plastic rather than glass. This is the opposite of in vivo, which indicates that the process takes place in a living organism. Under the right conditions the cells can divide to produce new cells that are exact copies of the original cells. In this way, the cell lines can be kept indefinitely. Many cell lines can also be frozen for future use.

Adult stem cells are already being used clinically, for instance to repair damaged heart tissue and to promote bone healing. The UM stem cell lines would probably not be used as a new treatment since they contain genetic defects. Their use is targeted more for research to study the diseases their genes encode. The availability of the new UM stem cell lines could provide valuable new opportunities for better understanding hemophilia B.

Share a Moment of Your Spectacular Life!

Submit your "action" photos! Share the good, the interesting, the fun and the funny! Matrix Health is compiling a 2013 calendar depicting the special moments in the lives of people with bleeding disorders. Be part of this opportunity to show our community that even with a bleeding disorder, life is spectacular! For more information, please visit **www.matrixhealthgroup.com** or contact Maria Vetter at: **mariavetter@matrixhealthgroup.com**

Never doubt that a small group of thoughtful, committed citizens can change the world; Indeed, it is the only thing that ever has.

~ Margaret Mead

Save the Date!

We are delighted to present The Coalition for Hemophilia B Factor Nine Family Meeting!
Saturday, September 22, 2012 10:00 AM – 4:00 PM

Renaissance Columbus Downtown Hotel
50 North Third Street; Columbus Ohio 43215
(614) 228-5050 (Hotel room rate for Friday September 21st is $129.00)
(Please do not book reservations until August 5th)
Registration is required online at www.coalitionforhemophiliab.org
Agenda Details will follow shortly!

Educational * Fun * Supportive! – Guaranteed!

WE LOOK FORWARD TO SEEING YOU!
The next meeting will be in Dallas, Texas at the end of October.
Details coming soon!

Survey Reminder

Please send in your survey responses! You input is valuable to the Coalition and helps us to address your overall concerns so that we can do our good work better FOR YOU! If you cannot find your copy please email Kim to receive another copy ASAP. Email Kim at **hemob@ix.netcom.com**

Comprehensive Health Education Services announces that registration is now OPEN for Inhibitor Family Camp, slated for Friday, September 28th thru Sunday, the 30th. Space is limited, and slots are filled on a first-come, first-served basis for those who qualify. To register, applicants must have an active inhibitor and be between the ages of 6-19. To distribute information about the program to any persons of interest, a printable brochure is available for your convenience. For additional information, please visit:

www.InhibitorFamilyCamp.org.

We are now on Facebook!
Visit us under
The Coalition for Hemophilia B

The Factor Nine Group moderated by Jill Lathrop is located on Facebook - search Hemophilia B Group

For back issues of **Factor Nine Newsletter** or for more information on research, please call or write to:
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